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## Personalized Cell Therapy for Diabetes

### Grant Award Details

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Personalized Cell Therapy for Diabetes

**Grant Type:** Therapeutic Translational Research Projects

**Grant Number:** TRAN1-08561-A

**Project Objective:** To optimize manufacturing, safety & efficacy testing of cell therapy product, and complete a Pre-IND meeting with the FDA.

**Investigator:**

<b>Name:</b>	Felicia Pagliuca
<b>Institution:</b>	Semma Therapeutics
<b>Type:</b>	PI

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**Disease Focus:** Diabetes, Metabolic Disorders

**Human Stem Cell Use:** iPS Cell

**Cell Line Generation:** iPS Cell

**Award Value:** \$597,333

**Status:** Closed

### Grant Application Details

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**Application Title:** Personalized Cell Therapy for Diabetes

**Public Abstract:****Translational Candidate**

Preclinical studies will develop patient specific stem cell-derived islets that secrete insulin & other islet hormones for regulation of blood sugar

**Area of Impact**

Genetically matched stem cell derived islets could provide treatment for diabetes without the need for immunosuppression or implantable devices.

**Mechanism of Action**

The stem cell-derived islets contain insulin-producing pancreatic  $\beta$  cells. These differentiated cells will be transplanted into patients who suffer from diabetes in order to replace the  $\beta$  cells that are missing or dysfunctional in the pancreatic islets of those patients. The stem cells used to generate these replacement islets will be genetically matched to the patient, enabling transplantation without long-term immunosuppression.

**Unmet Medical Need**

Millions suffer from Type 1 & Type 2 diabetes, which significantly impact quality of life and lead to serious complications. This proposal develops an alternative therapy with potential to transform the lives of those patients. First target is patients with insulin dependent, non-autoimmune diabetes

**Project Objective**

Pre-IND meeting with the FDA

**Major Proposed Activities**

- Production of the starting stem cell material from patients
- Optimize manufacturing of differentiated cell therapy product
- Preclinical safety and efficacy studies of the product

**Statement of Benefit to California:**

Many CA citizens are impacted by diabetes, including particularly high numbers with non-autoimmune, insulin dependent diabetes. This translational research aims to bring academic discoveries from lab of Doug Melton to the clinic, through generation of patient specific stem cell derived islets for transplant. Success in this program could have have immediate benefit to the California patients involved and would also lead the way to a new cell therapy broadly applicable to people with diabetes.

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